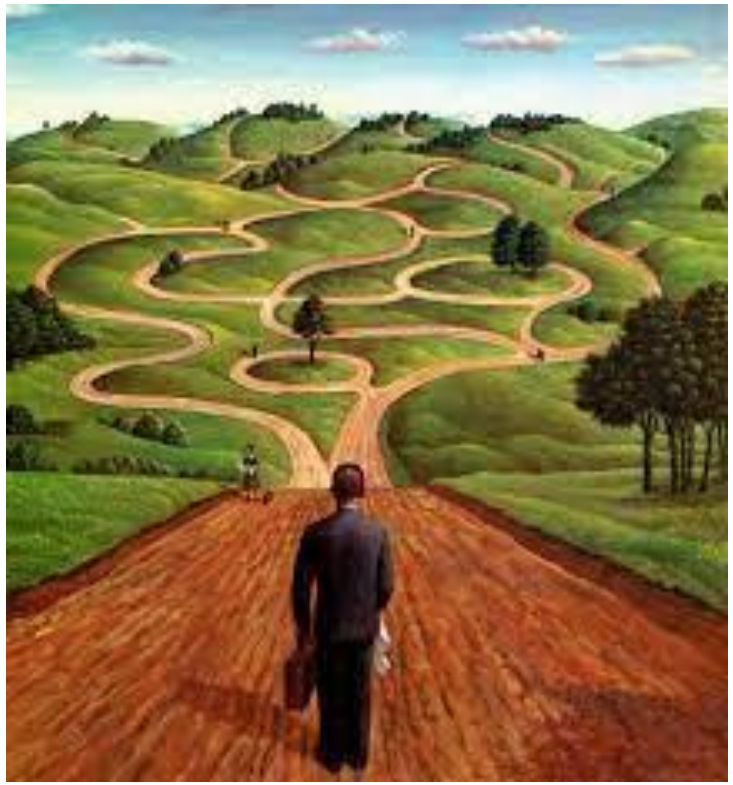
2023

International Patient Advocacy Group Meeting

REAL WORLD EVIDENCE & APAC
JULIE CINI –OAM



WHY COLLECT DATA?

- See who exists in your community
- Gives a long-term in-depth overview of the community
- Create some baseline data for your community.
- See an unmet need/ areas of concern within your community
- Gives real world evidence of what is happening in the life of someone living with the condition
- Gives creditability and validity to patient perspective.
- Informs your regulatory what is actually happening within the community

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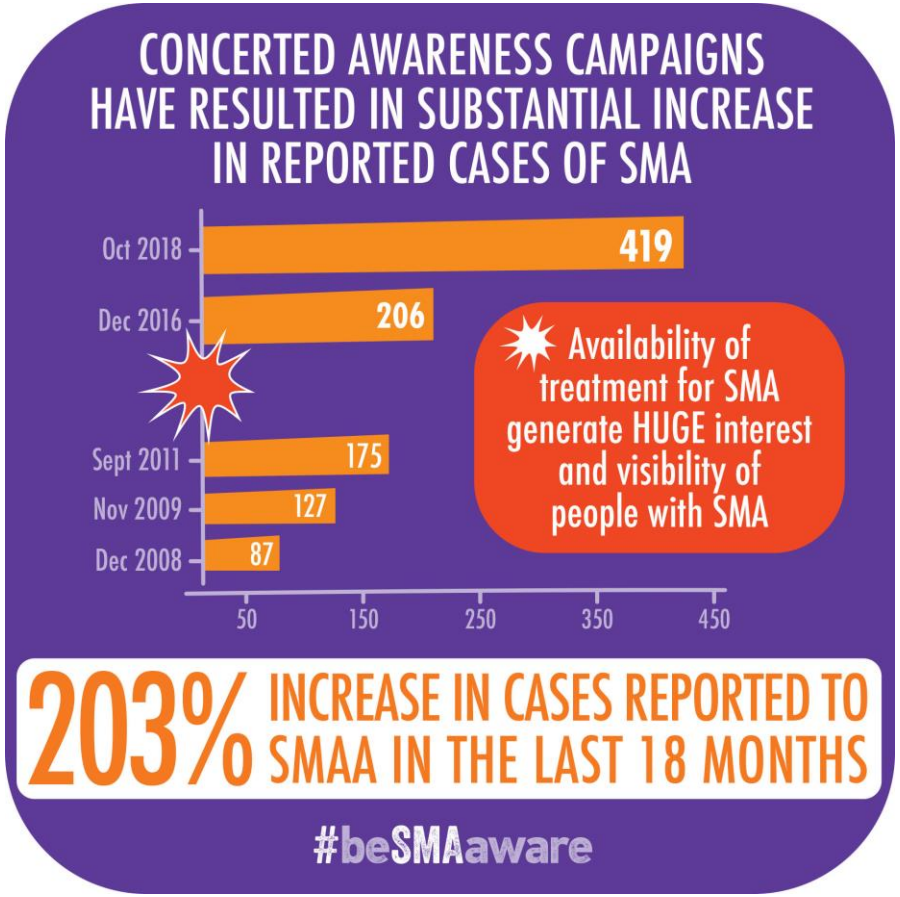
HOW LONG DO I NEED TO COLLECT DATA?



- **Start Early and collect often (every 12 months)**
- **Patterns and themes will develop within the data collected that you can report on**
- **Additional data can quickly be gathered as you don't need ethics approval**

WHAT DATA DO I NEED TO COLLECT?

- **Demographics- male/female, age, postcode**
- **Type (if any), age of onset, age of diagnosis**
- **Improvement/ deterioration**
- **Daily living skills achievable**
- **Carer hours required**
- **Quality of life questions relating to everyday living skills**
- **What the current situation looks like in your country.**
- **What would having access to treatment mean for your family**

ACCESS TO MEDICINE IS A MARATHON NOT A SPRINT!

- **Inform your community about the timeline of access to medicine in your country**
- **Not all medicines get through on a first submission**
- **Get in contact with a representative from your regulatory body in your country as to better understand how a medicine becomes commercially available.**
- **Work with your pharma company early! They shouldn't be coming to you last minute…..**

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POWER OF A STORY

- Encourage your families to pen their story
- Use these to capture the up's and downs within your community
- Regularly publish these stories to create awareness (media love good news stories)
- When advocating send these stories to your local politician (they are there to support you)

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DATA COLLECTION WORKS!

Approved in Australia in a 5 year time frame

- **2018 – first ever treatment for SMA reimbursed for people 18 years and younger**
- **2020 – MSAC approved carrier screening for SMA, Cystic Fibrosis and Fragile X (being implemented in November 2023)**
- **2021 –first oral treatment for SMA reimbursed for people 18 years and younger**
- **2021 –first gene therapy for SMA recommended for reimbursement for infants nine months and younger**
- **2021 – National newborn screening program recommended by the government (Available in all states except SA, TAS and half of Darwin)**
- **2022 – first gene therapy for SMA recommended in Australia**
- **2023 – first oral treatment recommended for adults over 19 years of age.**

ASIA PACIFIC


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